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Development of lead compound for Leber hereditary optic neuropathy

Curome Biosciences Co., Ltd.

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OPHTHALMOLOGY | **Lead**

Product Type	Small molecule drug
Indication	Leber Hereditary Optic Neuropathy (LHON)
Target	NADH:quinone oxidoreductase 1 (NQO1)
MoA(Mechanism of Action)	NQO1-based Oxidoreduction cycle (NOC) activator induces mitochondrial biosynthesis by activating SIRT1/AMPK/PGC-1α pathway by increasing intracellular nicotinamide adenine dinucleotide (NAD+) levels. In addition, NOC activator increases mitochondrial adenosine triphosphate (ATP) generation by donating electrons to mitochondrial complex III. By doing so, it can treat LHON caused by mitochondrial complex I deficiency.
Competitiveness	1. Best-in-Class 2. Competitor (Idebenone) was only approved for treatment of LHON under exceptional circumstance by the European Medicines Agency (EMA), but not approved by the U.S. Food and Drug Administration (FDA). 3.NOC activator is superior to competitor in improving LHON symptoms in animal model 4. NOC activator is a new synthetic compound, and a substance patent was applied for in 2023.
Development Stage	Lead
Route of Administration	Oral administration

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